Hematology Clinical Trials Portfolio

Sponsor ID	Protocol Title	Basic Eligibility	Status
Sickle Cell Disease			
CSEG101B2201	A Phase 2, Multicenter, Open-Label Study to Assess Appropriate Dosing and to Evaluate Safety of Crizanlizumab, with or without Hydroxyurea/ Hydroxycarbamide, in Sequential, Descending Age Groups of Pediatric Sickle Cell Disease Patients with Vaso-Occlusive Crisis	• Ages 2-18 • SCD (any genotype) • At least 1 documented VOC within 12 months • On stable dose of HU • Adequate renal/hepatic function, • Normal TCD • Negative pregnancy test, informed consent **Consider** Patients with any type of SCD who are	Group 1: (ages 12 to < 18yo) closed Group 2 (ages 6 to < 12yo) closed Group 3 (ages 6 months to <6 years): at least 8 participants ages 2 to <6 years and at least 6 participants ages 6 months to <24 months (only once the appropriate dose is confirmed in 2 to <6-year-old participants).
CSEG101A22401B	An Open-label, Multi-center, Phase IV, Rollover Study for Patients with Sickle Cell Disease who have Completed a Prior Novartis-Sponsored Crizanlizumab Study	Informed consent, SCD patient currently enrolled in a Novartis-sponsored study receiving crizanlizumab and has fulfilled all the requirements of the parent study, demonstration of compliance to the planned visit schedule in the parent study	Completed the crizanlizumab CSEG101B2201 study

GBT 440-032	A Phase 3, Randomized, Double-Blind, Placebo Controlled Study of Voxelotor (GBT440) in Pediatric Participants with Sickle Cell Disease and an Open-label Study in Infants with Sickle Cell Disease (HOPE Kids 2)	• Ages 2 to < 12 years • Any SCD genotype • At least 1 episode of VOC in the past 12 months. VOC defined as acute painful crisis which required prescription healthcare professional-instructed use of analgesics for moderate to severe • Stable HU dose (mg/kg) for at least 90 days for at least 90 days and with no anticipated need for dose adjustments or initiation during the study • Hb ≥ 5.5 g/dL ≤10.5 g/dL. • Adequate venous access • Female of childbearing age: use highly effective method of contraception and	Active, Enrolling
R01HL153386	Improving Scientific Rigor of Renal Clinical Endpoints for Sickle Cell Anemia- Sickle Cell Renal eGFR EquatioN (SCREEN) Study	• 5-50 years. • HbSS or HbSB0 thalassemia • Participants on any approved SCD medication is allowed to enroll • Participants on an investigational therapeutic study are allowed to enroll. **Consider** Any patient with sickle cell anemia. Four	Active, Enrolling
GRNDaD	A Prospective Study to Evaluate the Disease Status in Patients with Sickle Cell Disease: A Globin Regional Data and Discovery (GRNDaD) Network	Any form of SCD; Any age Consider Any patient with SCD can enroll in the national SCD registry. They can consent to biobanking of specimen for future research	Active, Enrolling
ITP			

H-42131 (ICON3)	A Phase 3 Study of Eltrombopag vs. Standard First-Line Management for Newly Diagnosed Immune Thrombocytopenia (ITP) in Children	• Age ≥ 1 year and < 18 years • Newly diagnosed ITP (<3 months from diagnosis(first abnormal platelet count) • Platelets < 30 x 10^9/L at screening • Requires pharmacologic treatment from the perspective of the treating clinician. • Treatment options include one the three standard therapies (IVG, corticosteroids or Anti-D immune globulin) Two groups 1.Upfront Treatment: - Patient within 10 days of ITP diagnosis who has not received previous treatment OR 2.Treatment failure -Patients who have failed standard management (observation or treatment with one or more first-line agents): -Failure of observation: No platelet recovery (>30 x 109/L) -Poor response to standard first-line agent (platelets remain <30 x 109/L) -Initial response to first line agent, but response wanes and platelets fall below 30	Active, Enrolling